Health outcomes research in an era of cost containment

Improving efficiency of research: decreasing costs, increasing quality

PART 2: INTERVENTIONS
MARCH 12, 2015

UNIVERSITÉ PARIS DESCARTES

SORBONNE PARIS CITÉ

Instituts thématiques Inserm
Institut national de la santé et de la recherche médicale

COLUMBIA UNIVERSITY EPIDEMIOLOGY
SCIENTIFIC SYMPOSIUM

COLUMBIA UNIVERSITY | MAILMAN SCHOOL of PUBLIC HEALTH EPIDEMIOLOGY

PART 2: INTERVENTIONS
MARCH 12, 2015

University Paris Descartes – INSERM
Centre de Recherche Épidémiologie et Statistique Sorbonne
Paris Cité (CRESS-UMR1153)
Paris, France

Dear colleagues,

Welcome to the symposium "Health outcomes research in an era of cost containment: Improving efficiency of research, decreasing costs, increasing quality". This event is co organized by Columbia University Mailman School of Public Health.

Today's symposium is particularly exciting because it is the first Europe-based event conceived as part of an active collaboration between Centre de Recherche Épidémiologie et Statistique Sorbonne Paris Cité and Columbia University (CRESS-UMR1153). Part 1, addressing observational studies, took place in New York one month ago on February 12. Today's symposium will address interventional studies.

We are excited about this timely event, striving to impact public discourse on innovative health outcomes research both in observational and interventions studies.

We would like to thank the Faculty members in New York and Paris who organized this symposium with us. Special acknowledgement is due to Dr. Sandro Galea, who was deeply involved in the design of the programme. Over the months developing this series, Sandro has been innovative, visionary, and a delight to work with. We are grateful to the current Chair of the Department of Epidemiology at Columbia, Neil W. Schluger, for seamless support. Acknowledgements go as well to the administrators whose hard work makes it all go smoothly.

Best regards,

Philippe Ravaud
Professor of Epidemiology
Paris Descartes University
Director of CRESS

AGENDA

8:30 – 9:00 BREAKFAST & INFORMAL GREETINGS

9:00 – 9:05 WELCOMING REMARKS

9:05 – 9:15 SYMPOSIUM OVERVIEW

9:15 – 10:45 PANEL 1: INNOVATIVE, MORE EFFICIENT TRIAL DESIGN

MODERATOR **Ying-Kueng Cheung, PhD**

No of 1 trials
Sunita Vohra, MD, MSc, FRCPC, FCAHS

Direct to patient trials
Steve R. Cummings, MD

Public led online trials
Amy Price, PhD

10:45 – 11:00 COFFEE BREAK

11:00 – 12:30 PANEL 2: SIMPLIFYING TRIALS OR IMPROVING OBSERVATIONAL STUDIES ACCURACY

MODERATOR **Sandro Galea MD, MPH, DrPH**

Changing trial metrics: The role of data management
Eric L. Eisenstein, DBA

Improving trial monitoring to reduce the costs
Tomasz Burzykowski, PhD

Analytic techniques to improve accuracy of observational studies
Jason Wright

12:30 – 13:30 LUNCH

13:30 – 15:00 PANEL 3: BLURRING THE LINES: USING OBSERVATIONAL STUDIES TO IMPROVE RECRUITMENTS IN RCTS

MODERATOR **Raphael Porcher, PhD**

Registry-based RCTs: A disruptive technology
Ole Fröbert, MD, PhD

A new trial design to speed RCTs: The I-SPY 2 trial
Donald Berry, MD, PhD

Strategies to improve recruitment in RCTs
Jonathan Craig, MBChB, DipCH, FRACP, M Med (Clin Epi), PhD

15:00 – 16:30 PANEL 4: BETTER REPORTING AND DATA SHARING FOR BETTER TRIAL RETURN ON INVESTMENT

MODERATOR **Alfred I. Neugut, MD, PhD, MPH**

Interventions to improve the quality of reporting and posting of results
Isabelle Boutron, PhD

Sharing clinical trial data on patient level
Martin Posch

16:30 - 16:45 CLOSING REMARKS

HOSTS

PHILIPPE RAVAUD, MD, PhD

Professor of Epidemiology, Paris Descartes University (France)

Adjunct Professor of Epidemiology, Columbia University's Mailman School of Public Health

Philippe Ravaud is a professor of epidemiology at Paris Descartes University and adjunct professor of epidemiology at the Mailman School of Public Health (Columbia University, New York City); director of the INSERM Epidemiology and Biostatistics Research Center, Sorbonne Paris Cité (INSERM UMR 1153); director of the Centre of Epidemiology at Hotel-Dieu; director of the French Cochrane Centre; and director of the French EQUATOR center. For more than 15 years, his primary focus has been evaluating and developing methodological research to assess treatments for patients with chronic diseases. His research activities during the last years have been structured around the following 4 main themes: evaluation of nonpharmacological treatments, development and validation of endpoints, meta-analyses and network meta-analyses, and research on research. His publication record includes more than 340 peer-reviewed papers, including more than 35 papers in the "big 6 journals" (New England Journal of Medicine, Lancet, JAMA, Annals of Internal Medicine, British Medical Journal and PLOS Medicine).

ALFRED I. NEUGUT, MD, PhD, MPH

Myron M. Studner Professor of Cancer Research of Medicine, New York Presbyterian Hospital and Columbia University Medical Center, College of Physicians and Surgeons

Associate Director for Population Sciences, Herbert Irving Comprehensive Cancer Center

Co-Director, Cancer Prevention Program, New York Presbyterian Hospital

Dr. Neugut is a medical oncologist, cancer epidemiologist, and health outcomes researcher. He did the first case-control study of adenomatous polyps, the precursor for colorectal cancer, and identified risk factors, including diet, obesity, asbestos, family history, tobacco and alcohol, the first such studies. He was first to suggest, in 1988, that colonoscopy be used for routine screening. Dr. Neugut also did>40 paperson multiple primary cancers, most importantly that breast cancer radiotherapy increased risk of subsequent lung and esophageal cancer, especially among smokers. He is co-PI of the Long Island Breast Cancer Study which generated >100 papers. Recently he has focused on quality of care in cancer. He found that not initiating adjuvant chemotherapy or hormonal therapy, when clinically indicated, is uncommon, but early discontinuation of adjuvant chemotherapy for colorectal and breast cancers is common and adversely affects survival. He also showed that higher co-payments are associated with non-adherence to adjuvant hormonal therapy.

ATLANTIC ALLIANCE FOR PUBLIC HEALTH

MOÏSE DESVARIEUX, MD, PHD

Associate Professor of Epidemiology, Columbia University

Directeur de Recherche, INSERM Epidemiology and Biostatistics Research Center, Sorbonne Paris Cité, (INSERM UMR 1153)

Director, Atlantic Alliance for Public Health

Dr. Desvarieux's work on chronic disease built on his initial background on infectious disease. Over the last 15 years, he has been continuously funded by the US National Institutes of Health (NIH) or European agencies as PI of large national research grants with cardiovascular outcomes, particularly focusing on the interface of chronic infections, inflammation and chronic disease and relevant methods. He has also led large international consortia on the subject. His work has been published in the Lancet, Circulation, Stroke, JAHA, and other leading journals in the field. In 2005, he was awarded a Chair of Excellence from the French National Institute of Health and Medical Research (INSERM) to coordinate a consortium of collaborating cohorts in Europe, US and Asia on the subject; and received the Leadership in Research award from the Friends of the National Institute of Dental and Craniofacial Research in Washington the same year. He has fostered the collaboration between France and Columbia University's Mailman School of Public Health over the last 7 years, leading to active and expanding agreements or collaborations with EHESP, the University of Paris-SPC Center for Epidemiology and Biostatistics, INSERM and the creation of the Atlantic Alliance for Public Health, for which he received the Dean's Leadership Award in 2010.

PARTICIPANTS

DONALD BERRY, PhD

Professor, Department of Biostatistics, University of Texas M.D. Anderson Cancer Center

A principal focus of Dr. Berry's research is the use of biomarkers in cancer and other diseases for learning which patients benefit from which therapies, based on -omics and phenotype. In particular, he designed and is a co-PI of I-SPY 2 www.ispy2.org, a Bayesian adaptive platform clinical trial in high-risk early breast cancer whose goal is matching experimental therapies with patient subsets that are defined by tumor molecular characteristics. Through Berry Consultants, LLC he has designed many innovative clinical trials in all therapeutic areas for pharmaceutical and medical device companies, for NIH cooperative groups, and for international consortia. He serves on the PDQ Screening and Prevention Board of the National Cancer Institute for which he received the National Institutes of Health Award of Merit in 2010. Dr. Berry is the author of several books on statistical methodology and over 400 publications, including first-authored articles in major medical journals. In 2014 he was named a Thomson Reuters Highly Cited Researcher in Clinical Medicine and listed as one of The World's Most Influential Scientific Minds in ScienceWatch.com.

ISABELLE BOUTRON, MD, PhD

Professor of Epidemiology, University Paris Descartes

Deputy Director, French EQUATOR center

Co-convenor, Cochrane Bias Methods group

Dr. Boutron's research activities mainly focus on internal and external validity of randomized and non-randomized studies evaluating interventions, the methodological issues when evaluating nonphamacologic treatments, and the dissemination and interpretation of research results. She particularly developed the concept of « spin » defined as the distortion of the presentation and interpretation of results in the field of biomedical research. She has published 89 peer-reviewed articles, 59 as first, second or last author.

TOMASZ BURZYKOWSKI, PhD

Professor of Biostatistics at Hasselt University (Belgium)

Vice-President of Research, International Drug Development Institute (IDDI)

Dr. Burzykowski has published methodological work on various topics, including survival analysis, meta-analysis, and validation of surrogate endpoints. Prior to joining UHasselt and IDDI, he worked at the Institute of Oncology in Warsaw (Poland). He has held visiting-professor positions at Karolinska Institute (Sweden), Warsaw University (Poland), Technical University of Warsaw (Poland), and Medical University of Bialystok (Poland). He is a co-author of two books. He is also a co-author of numerous papers presenting applications of statistical methods to clinical data in different disease areas. He currently serves as an Associate Editor of Biometrics and Pharmaceutical Statistics.

JONATHAN CRAIG, MBCHB, DIPCH, FRACP, M MED (CLIN EPI), PhD

Professor of Clinical Epidemiology, School of Public Health

Associate Dean (Research), Sydney Medical School

Dr. Craig is an internationally renowned paediatric nephrologist and clinical epidemiologist whose research aims to improve health care and clinical outcomes particularly in the areas of chronic kidney disease (CKD) and more broadly in child health through rigorous analysis of the evidence for commonly-used and novel interventions in CKD, identifying gaps/inconsistency in the evidence, conducting methodologically-sound clinical trials, and application of the research findings to clinical practice and policy. Professor Craig has contributed significantly toward effecting profound changes to the clinical research landscape in CKD. His methods research has led to increased and improved clinical research in CKD, systematic reviews that have informed the design of trials and content of national and international guidelines, the development of evidence-based clinical practice guidelines, and identification of issues specific to children. He has led the formation of state, national and international networks to facilitate the conduct of high-quality, relevant trials in children. He has been a member of the KDIGO Executive and the WHO Advisory Group on Clinical Trials in Children. He serves on the Medicare Services Advisory Committee and on the Council of the Australia-New Zealand Society of Nephrology.

STEVEN R. CUMMINGS, MD

Director, San Francisco Coordinating Center

Emeritus Professor of Medicine and Epidemiology, University of California, San Francisco

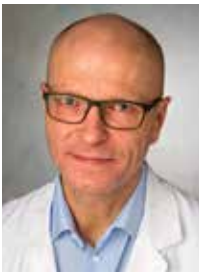
Senior Scientist, California Pacific Medical Center, Research Institute

ERIC L. EISENSTEIN, DBA

Associate Professor of Medicine, Duke University Medical Center

Dr. Cummings is the Founding Director of San Francisco Coordinating Center (SFCC), which leads and coordinates several large studies in women's health and osteoporosis. He co-founded the UCSF Training Program in Clinical Research, teaches research and trial methods, and has co-authored a standard textbook, "Designing Clinical Research" published by Wolters Kluwer | Lippincott Williams & Wilkins. Dr. Cummings founded 1747 (in 1999) and Mytrus (in 2010) to conduct internet-based clinical trials with novel e-consent systems. He served as "Innovator-in-Residence" at Genentech Roche in 2013-14 developing a successful method for radically simplifying protocols for clinical trials. He has published over 500 original articles about clinical research trials and aging and women's health. Dr. Cummings has been elected to the Institute of Medicine (IOM) of the U.S. National Academy of Sciences for his work in clinical research.

Dr. Eisenstein's major research focus is the economics of medical care and clinical research. He has led economic and quality of life studies of pharmaceutical, device and process of care interventions conducted alongside randomized clinical trials in cardiovascular, emergency, public health, and pulmonary medicine and vascular surgery. Dr. Eisenstein has a long-standing interest in health information technologies both as health care interventions and as tools for reducing the costs of clinical trials. In this regard, he co-directed a series of four population-based clinical trials that evaluated asynchronous clinical decision support systems as means for care coordination among 40,000 Medicaid patients. Dr. Eisenstein has led several studies that investigated the economics of phase III cardiovascular trials. These studies identified site-based costs (work performed by site personnel or site management) as the primary clinical trial cost drivers and highlighted monitoring as an area for cost reduction. This work provided support for large simple trial designs.



OLE FRÖBERT, MD, PhD

Adjunct Professor and Invasive Consultant Cardiologist, Örebro University Hospital, Sweden

Dr. Fröbert is an invasive consultant cardiologist at Örebro University Hospital, Sweden. He received his cardiology and research training in Denmark, Sweden, The Netherlands and the UK. His research and teaching focus on ischemic heart disease in a broad clinical perspective. Dr. Fröbert was primary investigator in the first registry - based randomized clinical trial - the 7000 patient TASTE trial. This novel and simple trial design has been termed a disruptive technology in clinical medicine. He is chairing or involved in a number of ongoing trials based on the same concept investigating diagnostic strategies, medical treatment and vaccination in patients with ischemic heart disease. Dr. Fröbert is a relentless advocate for originality, increased real value and reduced waste in medical research as reflected in the registry – based trial concept as well as his translational research on brown bears.

MARTIN POSCH

Professor of Medical Statistics, Medical University of Vienna

Head of the Section of Medical Statistics

Since September 2012 Martin Posch is professor of Medical Statistics at the Medical University of Vienna and head of the Section of Medical Statistics. Lastly, he worked as statistical expert at the European Medicines Agency (London, UK) in the Human Medicines Development and Evaluation sector, where he contributed to guideline development and the assessment of study designs. He has a PhD in Mathematics from the University of Vienna and was scientific assistant and associate professor at the Medical University of Vienna. His research interests are group sequential trials, adaptive designs and multiple testing, focusing on applications in clinical trials and Bioinformatics. Martin Posch serves as Associate Editor of Biometrics and Biometrical Journal.

AMY PRICE, PhD

Director, ThinkWell

SUNITA VOHRA, MD, MSc, FRCPC, FCAHS

Centennial Professor, Faculty of Medicine and School of Public Health, University of Alberta

Dr. Price's goal is build clear channels to propel evidence into practice by supplying the public, and those in low resource areas, with tools to make evidence-based healthcare choices. Responsible shared decision-making requires access to standardized and accurate shared knowledge. She and her team plan to engage, train and empower the public to plan, prioritize and take part in all aspects of research including the formation of online randomized controlled trials prioritised by the public and supported through expert methodological input. Her background in international relief work, clinical neurocognitive rehabilitation, service on the boards of multiple patient organizations and as a trauma survivor has equipped her with the flexible mindset to relate to all stakeholders and cultures.

A clinician scientist, Dr. Vohra is interested in advancing clinical research methods to help provide clinically relevant evidence. With regards to assessing effectiveness, Dr. Vohra has expertise in systematic reviews and pragmatic randomized controlled trial designs including cluster-controlled trials and N-of-1 trials. Dr. Vohra led the development of the CONSORT Extension for N-of-1 Trials (under review). With regards to safety, Dr. Vohra's methodological research is focused on improving the identification and reporting of harms in systematic reviews as well as in primary studies. Dr. Vohra is a Co-Convenor for the Cochrane Collaboration Adverse Effects Methods Group and has led the development of PRISMA Harms (in preparation). Dr. Vohra also leads population-based observational active surveillance studies to investigate the safety of popular health products and practices. Her work has revealed that active surveillance can improve harms identification and reporting many thousand fold over passive surveillance.

JASON WRIGHT

Chief, Division of Gynecologic Oncology

Sol Goldman Associate Professor of Obstetrics and Gynecology, Columbia University

Dr. Wright has published more than 170 peer reviewed scientific manuscripts as well as a number of textbooks and chapters. Dr. Wright's major research focus is comparative effectiveness and health services research. He has conducted a number of studies on the diffusion of novel drugs and technologies for cancer patients, surgical quality, and the management of complications in cancer patients. Dr. Wright' research has been funded by the National Cancer Institute, the Department of Defense, and the Gynecologic Cancer Foundation. Dr. Wright is an active member of the Society of Gynecologic Oncology, the American Society of Clinical Oncology, the American College of Surgeons and the American College of Obstetricians and Gynecologists.